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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

[Mark One]

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the Quarterly Period Ended June 30, 2018

OR

☐ **TRANSITION REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

Commission File No. 001-33057

CATALYST PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**76-0837053**
(State or other jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**
355 Alhambra Circle Suite 1250 Coral Gables, Florida	**33134**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (305) 420-3200

Indicate by checkmark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such report(s), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See definitions of "accelerated filer", "large accelerated filer", "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act (Check one):

Large accelerated filer	☐	Accelerated Filer	☒
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date 102,599,258 shares of common stock, $0.001 par value per share, were outstanding as of August 3, 2018.

CATALYST PHARMACEUTICALS, INC.

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CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS

	June 30, 2018 (unaudited)	December 31, 2017
ASSETS		
Current Assets:		
Cash and cash equivalents	$ 9,887,939	$ 57,496,702
Short-term investments	58,520,956	26,516,711
Prepaid expenses and other current assets	677,341	1,173,744
Total current assets	69,086,236	85,187,157
Investments	5,005,321	—
Property and equipment, net	187,131	191,385
Deposits	8,888	8,888
Total assets	$ 74,287,576	$ 85,387,430
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current Liabilities:		
Accounts payable	$ 922,593	$ 1,945,575
Accrued expenses and other liabilities	2,159,435	2,320,587
Total current liabilities	3,082,028	4,266,162
Accrued expenses and other liabilities, non-current	143,335	157,456
Total liabilities	3,225,363	4,423,618
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.001 par value, 5,000,000 shares authorized: none issued and outstanding at June 30, 2018 and December 31, 2017	—	—
Common stock, $0.001 par value, 150,000,000 shares authorized; 102,599,258 shares and 102,549,498 shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively	102,599	102,549
Additional paid-in capital	209,221,591	207,421,710
Accumulated deficit	(138,225,479)	(126,560,447)
Accumulated other comprehensive loss	(36,498)	—
Total stockholders' equity	71,062,213	80,963,812
Total liabilities and stockholders' equity	$ 74,287,576	$ 85,387,430

The accompanying notes are an integral part of these consolidated financial statements.

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS (unaudited)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2018	2017	2018	2017
Operating costs and expenses:				
Research and development	$ 3,704,824	$ 2,451,751	$ 6,963,866	$ 5,265,680
General and administrative	2,631,031	1,729,520	5,305,429	3,595,462
Total operating costs and expenses	6,335,855	4,181,271	12,269,295	8,861,142
Loss from operations	(6,335,855)	(4,181,271)	(12,269,295)	(8,861,142)
Other income, net	370,715	91,039	604,263	201,016
Change in fair value of warrants liability	—	210,331	—	(186,904)
Loss before income taxes	(5,965,140)	(3,879,901)	(11,665,032)	(8,847,030)
Provision for income taxes	—	—	—	—
Net loss	$ (5,965,140)	$ (3,879,901)	$ (11,665,032)	$ (8,847,030)
Net loss per share – basic and diluted	$ (0.06)	$ (0.05)	$ (0.11)	$ (0.11)
Weighted average shares outstanding – basic and diluted	102,596,446	83,905,827	102,577,005	83,441,650
Net loss	$ (5,965,140)	$ (3,879,901)	$ (11,665,032)	$ (8,847,030)
Other comprehensive loss:				
Unrealized gain (loss) on available-for-sale securities	(14,672)	—	(36,498)	—
Comprehensive loss	$ (5,979,812)	$ (3,879,901)	$ (11,701,530)	$ (8,847,030)

The accompanying notes are an integral part of these consolidated financial statements.

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY (unaudited)
For the six months ended June 30, 2018

	Preferred Stock	Common Stock	Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total
Balance at December 31, 2017	$ —	$102,549	$207,421,710	$(126,560,447)	$ —	$ 80,963,812
Issuance of common stock, net	—	3	10,546	—	—	10,549
Issuance of stock options for services	—	—	1,747,850	—	—	1,747,850
Exercise of stock options for common stock	—	47	41,485	—	—	41,532
Other comprehensive loss	—	—	—	—	(36,498)	(36,498)
Net loss	—	—	—	(11,665,032)	—	(11,665,032)
Balance at June 30, 2018	$ —	$102,599	$209,221,591	$(138,225,479)	$ (36,498)	$ 71,062,213

The accompanying notes are an integral part of these consolidated financial statements.

CATALYST PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS (unaudited)

	For the Six Months Ended June 30,	
	2018	2017
Operating Activities:		
Net loss	$ (11,665,032)	$ (8,847,030)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	16,191	25,915
Stock-based compensation	1,762,847	1,392,713
Change in fair value of warrants liability	—	186,904
(Increase) decrease in:		
Prepaid expenses and other current assets and deposits	496,403	426,386
Increase (decrease) in:		
Accounts payable	(1,022,982)	(240,831)
Accrued expenses and other liabilities	(175,273)	(23,921)
Net cash used in operating activities	(10,587,846)	(7,079,864)
Investing Activities:		
Purchases of property and equipment	(11,937)	—
Purchases of investments	(37,046,064)	(34,910)
Net cash provided by (used in) investing activities	(37,058,001)	(34,910)
Financing Activities:		
Payment of employee withholding tax related to stock-based compensation	(4,448)	—
Proceeds from exercise of warrants	—	1,805,437
Proceeds from exercise of stock options	41,532	—
Net cash provided by (used in) financing activities	37,084	1,805,437
Net increase (decrease) in cash and cash equivalents	(47,608,763)	(5,309,337)
Cash and cash equivalents - beginning of period	57,496,702	13,893,064
Cash and cash equivalents - end of period	$ 9,887,939	$ 8,583,727
Non-cash investing and financing activities:		
Unrealized gain (loss) on available-for-sale securities	$ (36,498)	$ —
Exercise of liability classified warrants for common stock	$ —	$ 309,130

The accompanying notes are an integral part of these consolidated financial statements.

CATALYST PHARMACEUTICALS, INC.

NOTES TO UNAUDITED CONSOLIDATED FINANCIAL STATEMENTS

1. Organization and Description of Business.

Catalyst Pharmaceuticals, Inc. (the Company) is a development-stage biopharmaceutical company focused on developing and commercializing innovating therapies for people with rare debilitating, chronic neuromuscular and neurological diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndromes (CMS), MuSK antibody positive myasthenia gravis (MuSK-MG), and infantile spasms.

Since inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, acquiring operating assets and raising capital. The Company's primary focus is on the development and commercialization of its drug candidates. The Company has incurred operating losses in each period from inception through June 30, 2018. The Company has been able to fund its cash needs to date through several public and private offerings of its common stock and warrants, through government grants, and through an investment by a strategic purchaser. See Note 10.

Capital Resources

While there can be no assurance, based on currently available information, the Company estimates that it has sufficient resources to support its operations for at least the next 12 months.

The Company may raise additional funds in the future, if required for its business, through public or private equity offerings, debt financings, corporate collaborations, governmental research grants or other means. The Company may also seek to raise new capital to fund additional product development efforts, even if it has sufficient funds for its planned operations. Any sale by the Company of additional equity or convertible debt securities could result in dilution to the Company's current stockholders. There can be no assurance that any required additional funding will be available to the Company at all or available on terms acceptable to the Company. Further, to the extent that the Company raises additional funds through collaborative arrangements, it may be necessary to relinquish some rights to the Company's drug candidates or grant sublicenses on terms that are not favorable to the Company. If the Company is not able to secure additional funding when needed, the Company may have to delay, reduce the scope of, or eliminate one or more research and development programs, which could have an adverse effect on the Company's business.

2. Basis of Presentation and Significant Accounting Policies.

a. INTERIM FINANCIAL STATEMENTS. The accompanying unaudited interim consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles (U.S. GAAP), and pursuant to the rules and regulations of the Securities and Exchange Commission (SEC) for reporting of interim financial information. Pursuant to such rules and regulations, certain information and note disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been omitted. The consolidated balance sheet as of December 31, 2017 included in this Form 10-Q was derived from the audited financial statements and does not include all disclosures required by U.S. GAAP.

In the opinion of management, the accompanying unaudited interim consolidated financial statements of the Company contain all adjustments (consisting of only normal recurring adjustments) necessary to present fairly the financial position of the Company as of the dates and for the periods presented. Accordingly, these consolidated statements should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2017 included in the 2017 Annual Report on Form 10-K filed by the Company with the SEC. The results of operations for the six months ended June 30, 2018 are not necessarily indicative of the results to be expected for any future period or for the full 2018 fiscal year.

2. Basis of Presentation and Significant Accounting Policies (continued).

b. PRINCIPLES OF CONSOLIDATION. The consolidated financial statements include the Company's accounts and those of its wholly-owned subsidiary Catalyst Pharmaceuticals Ireland, Ltd. ("Catalyst Ireland"). All intercompany accounts and transactions have been eliminated in consolidation. Catalyst Ireland was organized in 2017.

c. USE OF ESTIMATES. The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

d. CASH AND CASH EQUIVALENTS. The Company considers all highly liquid instruments purchased with an original maturity of three months or less to be cash equivalents. Cash equivalents consist mainly of money market funds. The Company has substantially all of its cash and cash equivalents deposited with one financial institution. These amounts at times may exceed federally insured limits.

e. INVESTMENTS. The Company invests in high credit-quality funds in order to obtain higher yields on its cash available for investments. At June 30, 2018, investments consisted of a short-term bond fund and U.S. Treasuries. At December 31, 2017, investments consisted of a short-term bond fund. Such investments are not insured by the Federal Deposit Insurance Corporation.

Short-term bond fund

The short-term bond fund is classified in trading securities. Trading securities are recorded at fair value based on the closing market price of the security. For trading securities, the Company recognizes realized gains and losses and unrealized gains and losses to earnings. At June 30, 2018 and December 31, 2017, the only investment classified as trading securities was the short-term bond fund. Unrealized gain (loss) on trading securities was $29,431 and ($29,430), respectively, for the three and six months ended June 30, 2018, and $0 and $29,430 for the three and six months ended June 30, 2017 and is included in other income, net in the accompanying consolidated statements of operations.

U.S. Treasuries

U.S. Treasuries are classified as available-for-sale securities. The Company classifies available-for-sale securities with stated maturities of greater than three months and less than one year from the date of purchase as short-term investments. Available-for-sale securities with stated maturities greater than one year are classified as non-current investments in the accompanying consolidated balance sheets. The Company records available-for-sale securities at fair value with unrealized gains and losses reported in accumulated other comprehensive income (loss) in stockholders' equity. Realized gains and losses are included in other income, net and are derived using the specific identification method for determining the cost of securities sold. Interest income is recognized when earned and is included in other income, net in the consolidated statements of operations. The Company recognizes a charge when the declines in the fair value below the amortized cost basis of its available-for-sale securities are judged to be other-than-temporary. The Company considers various factors in determining whether to recognize an other-than-temporary charge, including whether the Company intends to sell the security or whether it is more likely than not that the Company would be required to sell the security before recovery of the amortized cost basis. The Company has not recorded any other than temporary impairment charges on its available-for-sale securities. See Note 4.

f. PREPAID EXPENSES AND OTHER CURRENT ASSETS. Prepaid expenses and other current assets consist primarily of prepaid research fees, prepaid pre-commercialization expenses, prepaid insurance and prepaid subscription fees. Prepaid research fees consist of advances for the Company's product development activities, including drug manufacturing, contracts for pre-clinical studies, clinical trials and studies, regulatory affairs and consulting. Such advances are recorded as expense as the related goods are received or the related services are performed.

2. Basis of Presentation and Significant Accounting Policies (continued).

g. FAIR VALUE OF FINANCIAL INSTRUMENTS. The Company's financial instruments consist of cash and cash equivalents, short-term investments, accounts payables, accrued expenses and other liabilities, and warrants liability. At June 30, 2018 and December 31, 2017, the fair value of these instruments approximated their carrying value.

h. FAIR VALUE MEASUREMENTS. Current Financial Accounting Standards Board (FASB) fair value guidance emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Therefore, a fair value measurement should be determined based on the assumptions that market participants would use in pricing the asset or liability. As a basis for considering market participant assumptions in fair value measurements, current FASB guidance establishes a fair value hierarchy that distinguishes between market participant assumptions based on market data obtained from sources independent of the reporting entity (observable inputs that are classified within Levels 1 and 2 of the hierarchy) and the reporting entity's own assumptions that it believes market participants would use in pricing assets or liabilities (unobservable inputs classified within Level 3 of the hierarchy).

Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date. Level 2 inputs are inputs other than quoted prices included in Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs may include quoted prices for similar assets and liabilities in active markets, as well as inputs that are observable for the asset or liability (other than quoted prices), such as interest rates, foreign exchange rates, and yield curves that are observable at commonly quoted intervals. Level 3 inputs are unobservable inputs for the asset or liability, which are typically based on an entity's own assumptions, as there is little, if any, related market activity. In instances where the determination of the fair value measurement is based on inputs from different levels of the fair value hierarchy, the level in the fair value hierarchy within which the entire fair value measurement falls is based on the lowest level input that is significant to the fair value measurement in its entirety. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment, and considers factors specific to the asset or liability.

		Fair Value Measurements at Reporting Date Using		
	Balances as of June 30, 2018	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$ 9,496,648	$ 9,496,648	$ —	$ —
Short-term investments:				
Short-term bond fund	$26,530,474	$ 26,530,474	$ —	$ —
U.S. Treasuries	$31,990,482	$ —	$31,990,482	$ —
Investments:				
U.S. Treasuries	$ 5,005,321	$ —	$ 5,005,321	$ —

	Balances as of December 31, 2017	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash and cash equivalents:				
Money market funds	$56,820,688	$ 56,820,688	$ —	$ —
Short-term investments:				
Short-term bond fund	$26,516,711	$ 26,516,711	$ —	$ —

2. Basis of Presentation and Significant Accounting Policies (continued).

i. WARRANTS LIABILITY. In October 2011, the Company issued 1,523,370 warrants (the 2011 warrants) to purchase shares of the Company's common stock in connection with a registered direct offering. During the period that the 2011 warrants were outstanding, the Company accounted for these warrants as a liability measured at fair value due to a provision included in the warrants agreement that provided the warrants holders with an option to require the Company (or its successor) to purchase their warrants for cash in an amount equal to their Black-Scholes Option Pricing Model (the Black-Scholes Model) value, in the event that certain fundamental transactions, as defined, occurred. The fair value of the warrants liability was estimated using the Black-Scholes Model which required inputs such as the expected term of the warrants, share price volatility and risk-free interest rate. These assumptions were reviewed on a quarterly basis and changes in the estimated fair value of the outstanding warrants were recognized each reporting period in the "Change in fair value of warrants liability" line in the consolidated statement of operations. All unexercised 2011 warrants expired on May 2, 2017. See Note 3.

j. STOCK-BASED COMPENSATION. The Company recognizes expense in the consolidated statements of operations for the fair value of all stock-based payments to employees, directors, scientific advisors and consultants, including grants of stock options and other share-based awards. For stock options, the Company uses the Black-Scholes option valuation model, the single-option award approach, and the straight-line attribution method. Using this approach, compensation cost is amortized on a straight-line basis over the vesting period of each respective stock option, generally one to three years. Forfeitures are recognized as a reduction of stock-based compensation expense as they occur.

As of June 30, 2018, there were outstanding stock options to purchase 7,719,166 shares of common stock, of which stock options to purchase 4,096,662 shares of common stock were exercisable as of June 30, 2018.

For the three and six-month periods ended June 30, 2018 and 2017, the Company recorded stock-based compensation expense as follows:

	Three months ended June 30,		Six months ended June 30,	
	2018	2017	2018	2017
Research and development	$285,625	$223,552	$ 579,940	$ 429,904
General and administrative	505,882	415,017	1,182,907	962,809
Total stock-based compensation	$791,507	$638,569	$1,762,847	$1,392,713

k. COMPREHENSIVE INCOME (LOSS). U.S. GAAP require that all components of comprehensive income (loss) be reported in the financial statements in the period in which they are recognized. Comprehensive income (loss) is net income (loss), plus certain other items that are recorded directly into stockholders' equity. The Company's comprehensive loss is shown on the Consolidated Statements of Operations and Comprehensive Loss for the three and six months ended June 30, 2018 and is comprised of net unrealized losses on the Company's available-for-sale securities. For December 31, 2017 and all prior periods, the Company's net loss equaled comprehensive loss, since the Company had no items which were considered other comprehensive income (loss).

2. Basis of Presentation and Significant Accounting Policies (continued).

l. NET LOSS PER SHARE. Basic loss per share is computed by dividing net loss for the period by the weighted average number of common shares outstanding during the period. The calculation of basic and diluted net loss per share is the same for all periods presented, as the effect of potential common stock equivalents is anti-dilutive due to the Company's net loss position for all periods presented. The potential shares, which are excluded from the determination of basic and diluted net loss per share as their effect is anti-dilutive, are as follows:

	June 30,	
	2018	2017
Options to purchase common stock	7,719,166	6,090,000
Warrants to purchase common stock	—	675,000
Unvested restricted stock	—	26,667
Potential equivalent common stock excluded	7,719,166	6,791,667

Potentially dilutive options to purchase common stock as of June 30, 2018 have exercise prices ranging from $0.79 to $4.64. Potentially dilutive options to purchase common stock as of June 30, 2017 had exercise prices ranging from $0.47 to $4.64. Potentially dilutive warrants to purchase common stock as of June 30, 2017 had an exercise price of $2.08 and expired in August 2017.

m. RECENTLY ISSUED ACCOUNTING STANDARDS. In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*, which requires an entity to recognize assets and liabilities arising from a lease for both financing and operating leases. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The Company's only significant lease is its facility lease, which expires on November 30, 2022. The Company anticipates recognition of an additional asset and corresponding liability related to its facility lease on the consolidated balance sheet. This standard will have a material impact on the Company's consolidated financial statements.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation – Stock Compensation (Topic 718): Scope of Modification Accounting* to clarify when to account for a change to the terms or conditions of a share-based payment award as a modification. Under this new guidance, modification accounting is required if the fair value, vesting conditions, or classification of the award changes as a result of the change in terms or conditions. ASU 2017-09 is effective for all entities for annual reporting periods beginning after December 15, 2017, including interim reporting periods within each annual reporting period, applied prospectively on or after the effective date. The Company adopted this standard in the first quarter of 2018. The adoption of this standard did not have a material impact on the Company's consolidated financial statements.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation - Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* that largely aligns the accounting for share-based payment awards issued to employees and nonemployees. Under the ASU, most of the guidance on such payments to nonemployees would be aligned with the requirements for share-based payments granted to employees. ASU 2018-07 is effective for all entities for annual reporting periods beginning after December 15, 2018, including interim reporting periods within each annual reporting period, with early adoption permitted. The Company is currently evaluating the impact this accounting standard will have on its consolidated financial statements.

n. RECLASSIFICATIONS. Certain prior year amounts in the consolidated financial statements have been reclassified to conform to the current year presentation.

3. Warrants Liability, at Fair Value.

2011 Warrants

The Company allocated approximately $1.3 million of proceeds from its October 2011 registered direct offering to the fair value of common stock purchase warrants issued in connection with the offering that were classified as a liability (the 2011 warrants). The 2011 warrants were classified as a liability because of provisions in such warrants that allowed for the net cash settlement of such warrants in the event of certain fundamental transactions (as defined in the warrant agreement). During the period that the 2011 warrants were outstanding, the valuation of the 2011 warrants was determined using the Black-Scholes Model. This model uses inputs such as the underlying price of the shares issued when the warrant is exercised, volatility, risk free interest rate and expected life of the instrument. The Company had determined that the 2011 warrants liability should be classified within Level 3 of the fair value hierarchy by evaluating each input for the Black-Scholes Model against the fair value hierarchy criteria and using the lowest level of input as the basis for the fair value classification. There are six inputs: closing price of the Company's common stock on the day of evaluation; the exercise price of the warrants; the remaining term of the warrants; the volatility of the Company's common stock; annual rate of dividends; and the risk-free rate of return. Of those inputs, the exercise price of the warrants and the remaining term were readily observable in the warrants agreement. The annual rate of dividends was based on the Company's historical practice of not granting dividends. The closing price of the Company's common stock would fall under Level 1 of the fair value hierarchy as it is a quoted price in an active market. The risk-free rate of return was a Level 2 input, while the historical volatility was a Level 3 input in accordance with the fair value accounting guidance. Since the lowest level input was a Level 3, the Company determined the 2011 warrants liability were most appropriately classified within Level 3 of the fair value hierarchy. This liability was subject to a fair value mark-to-market adjustment each reporting period. All unexercised 2011 warrants expired on May 2, 2017.

The following table rolls forward the fair value of the Company's warrants liability activity for the three and six-month periods ended June 30, 2017:

	Three months ended June 30, 2017	Six months ended June 30, 2017
Fair value, beginning of period	$ 519,461	$ 122,226
Issuance of warrants	—	—
Exercise of warrants	(309,130)	(309,130)
Change in fair value	(210,331)	186,904
Fair value, end of period	$ —	$ —

On May 2, 2017, the outstanding and unexercised 2011 warrants expired. During both the three and six months ended June 30, 2017, 613,913 of the 2011 warrants were exercised, with proceeds of $798,087 to the Company.

4. Investments.

Available-for-sale investments by security type were as follows:

	Amortized cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
At June 30, 2018:				
U.S. Treasuries - ST	$32,007,257	$ —	$ (16,775)	$31,990,482
U.S. Treasuries - LT	5,025,044	—	(19,723)	5,005,321
Total	$37,032,301	$ —	$ (36,498)	$36,995,803

At December 31, 2017, the Company did not have any available-for-sale securities.

4. Investments (continued).

In accordance with FASB ASC Topic 320, "Investments – Debt and Equity Securities", or ASC 320, the Company has classified its U.S. Treasuries as available-for-sale securities with secondary or resale markets, and, as such, they are reported at fair value with unrealized gain and losses included in comprehensive loss in stockholders' equity and realized gains and losses, included in other income, net. There were no realized gains or losses from available-for-sale securities for the three or six months ended June 30, 2018 or 2017.

Certain U.S. Treasuries at June 30, 2018 had fair values less than their amortized costs and, therefore, contained unrealized losses. Given that the Company has no intent to sell the U.S. Treasuries until a recovery of its fair value, which may be at maturity, and there are no current requirements to sell any of these securities, the Company did not consider these investments to be other-than-temporarily impaired as of June 30, 2018. The Company anticipates full recovery of amortized costs with respect to these investments at maturity. The duration of time the U.S. Treasuries have been in a continuous unrealized loss position as of June 30, 2018 was less than 6 months.

The estimated fair values of available-for-sale securities at June 30, 2018, by contractual maturity, are summarized as follows:

	June 30, 2018
Due in one year or less	$31,990,482
Due after one year but within two years	5,005,321
	$36,995,803

5. Prepaid Expenses and Other Current Assets.

Prepaid expenses and other current assets consist of the following:

	June 30, 2018	December 31, 2017
Prepaid research fees	$ 211,124	$ 388,977
Prepaid insurance	306,689	638,139
Prepaid pre-commercialization fees	8,925	65,000
Prepaid subscription fees	69,068	23,347
Prepaid rent	20,828	—
Other	60,707	58,281
Total prepaid expenses and other current assets	$ 677,341	$ 1,173,744

6. Property and Equipment, net.

Property and equipment, net consists of the following:

	June 30, 2018	December 31, 2017
Computer equipment	$ 27,915	$ 27,915
Furniture and equipment	181,868	169,931
Leasehold improvements	152,708	152,708
	362,491	350,554
Less: Accumulated depreciation	(175,360)	(159,169)
Total property and equipment, net	$ 187,131	$ 191,385

Depreciation expense was $8,176 and $16,191, respectively, for the three and six-month periods ended June 30, 2018 and $12,957 and $25,915, respectively for the three and six-month periods ended June 30, 2017.

7. Accrued Expenses and Other Liabilities.

Accrued expenses and other liabilities consist of the following:

	June 30, 2018	December 31, 2017
Accrued preclinical and clinical trial expenses	$ 1,251,568	$ 970,649
Accrued professional fees	338,098	227,457
Accrued compensation and benefits	232,847	821,935
Accrued license fees	305,000	252,500
Deferred rent and lease incentive	27,184	24,011
Other	4,738	24,035
Current accrued expenses and other liabilities	2,159,435	2,320,587
Deferred rent and lease incentive - non-current	143,335	157,456
Non-current accrued expenses and other liabilities	143,335	157,456
Total accrued expenses and other liabilities	$ 2,302,770	$ 2,478,043

8. Commitments and Contingencies.

a. LICENSE AGREEMENT WITH NORTHWESTERN UNIVERSITY. On August 27, 2009, the Company entered into a license agreement with Northwestern University (Northwestern), under which it acquired worldwide rights to commercialize new GABA aminotransferase inhibitors and derivatives of vigabatrin that have been discovered by Northwestern. Under the terms of the license agreement, Northwestern granted the Company an exclusive worldwide license to certain composition of matter patents related to the new class of inhibitors and a patent application relating to derivatives of vigabatrin. The Company has identified and designated the lead compound under this license as CPP-115.

Under the license agreement with Northwestern, the Company is responsible for continued research and development of any resulting product candidates. As of June 30, 2018, the Company has paid $424,885 in connection with the license and has accrued license fees of $305,000 in the accompanying June 30, 2018 consolidated balance sheet for expenses, maintenance fees and milestones. In addition, the Company is obligated to pay certain milestone payments in future years relating to clinical development activities with respect to CPP-115, and royalties on any products resulting from the license agreement, if the Company does not cancel the license agreement. The next milestone payment of $300,000 is due on the earlier of successful completion of the first Phase 3 clinical trial for CPP-115 or August 27, 2018.

Recently, the Company became aware that certain patents granted to Northwestern to a new GABA aminotransferase inhibitor and derivative of vigabatrin were derived from CPP-115. As such, it is the Company’s position that Northwestern is currently in breach of the license agreement based on its failure to transfer these new patent rights to the Company. Discussions to resolve this dispute are ongoing. There can be no assurance as to the outcome of this matter, and, if the matter is not amicably resolved, the Company intends to pursue its rights under its license agreement.

b. LICENSE AGREEMENT WITH NEW YORK UNIVERSITY AND THE FEINSTEIN INSTITUTE FOR MEDICAL RESEARCH. On December 13, 2011, the Company entered into a license agreement with New York University (NYU) and the Feinstein Institute for Medical Research (FIMR) under which it acquired worldwide rights to commercialize GABA aminotransferase inhibitors in the treatment of Tourette’s Disorder. The Company is obligated to pay certain milestone payments in future years relating to clinical development activities and royalties on any products resulting from the license agreement.

8. Commitments and Contingencies (continued).

c. LICENSE AGREEMENT WITH BIOMARIN. On October 26, 2012, the Company entered into a license agreement with BioMarin Pharmaceutical, Inc. (BioMarin) for the North American rights to Firdapse®.

Under the License Agreement, the Company has agreed to pay: (i) royalties to BioMarin for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in the license agreement) in North America for any calendar year for sales up to $100 million, and 10% of net sales in North America in any calendar year in excess of $100 million; and (ii) royalties to the third-party licensor of the rights sublicensed to the Company for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in the license agreement between BioMarin and the third-party licensor) in any calendar year.

Additionally, the Company's license agreement with BioMarin requires the Company to pay certain milestone payments that BioMarin is obligated to pay to both a third-party licensor of the rights that have been sublicensed to the Company and to the former stockholders of Huxley Pharmaceuticals ("Huxley") under an earlier stock purchase agreement between BioMarin and the former Huxley stockholders.

With respect to the third party licensor of the rights that have been sublicensed to the Company, the Company has agreed to pay: (i) $150,000 upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS (which amount was paid during the second quarter of 2018 after acceptance by the FDA of the Company's NDA for Firdapse® for LEMS), and (ii) approximately $3.0 million of which will be due upon the upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (which milestone payment has not yet been earned).

With respect to the former Huxley stockholders, the Company had agreed that it would pay the following milestone payments if either of the following milestones were satisfied before April 20, 2018: (i) $2,425,000 upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS, and (ii) approximately $4,200,000 of which was to be paid upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS. Since neither of these milestones were met on or before April 20, 2018, these milestone payments were never earned. However, prior to April 20, 2018, the Company was advised that the former Huxley stockholders intended to take legal action against BioMarin and the Company seeking payment of the milestone payments due to them even if the milestones were achieved after their expiration date (April 20, 2018). While the Company disputed its obligation to pay either of the above described milestone payments if these milestones were achieved after April 20, 2018, in an agreement which was executed and became effective on July 26, 2018, the Company, BioMarin and the former Huxley stockholders agreed to amicably resolve this matter. As part of the settlement, and without admitting any liability, the Company agreed to pay the former Huxley stockholders two new milestone payments in lieu of the milestone payments described above: (i) a $1.0 million milestone payment due upon execution of the settlement agreement, and (ii) a $1.0 million payment due on the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (which milestone payment has not yet been earned). Further, as part of the settlement agreement, the Company, BioMarin and the former Huxley stockholders entered into mutual releases regarding these matters. See Note 12.

The Company also agreed to share in the cost of certain post-marketing studies being conducted by BioMarin, and, as of June 30, 2018, the Company had paid BioMarin $3.8 million related to expenses in connection with Firdapse® studies and trials.

8. Commitments and Contingencies (continued).

d. AGREEMENTS FOR DRUG DEVELOPMENT, PRE-CLINICAL AND CLINICAL STUDIES. The Company has entered into agreements with contract manufacturers for the manufacture of drug and study placebo for the Company's trials and studies, with contract research organizations (CRO) to conduct and monitor the Company's trials and studies and with various entities for laboratories and other testing related to the Company's trials and studies. The contractual terms of the agreements vary, but most require certain advances as well as payments based on the achievement of milestones. Further, these agreements are cancellable at any time, but obligate the Company to reimburse the providers for any time or costs incurred through the date of termination.

9. Income Taxes.

The Company is subject to income taxes in the U.S. federal jurisdiction and various states jurisdictions. Tax regulations within each jurisdiction are subject to the interpretation of the related tax laws and regulations and require significant judgment to apply. The Company is not subject to U.S. federal, state and local tax examinations by tax authorities for any years before 2014. If the Company were to subsequently record an unrecognized tax benefit, associated penalties and tax related interest expense would be reported as a component of income tax expense.

The Company's net deferred tax asset has a 100% valuation allowance at June 30, 2018 and December 31, 2017 as the Company believes that it is more likely than not that the deferred tax asset will not be realized.

10. Stockholders' Equity.

2016 Shelf Registration Statement

On December 23, 2016, the Company filed a shelf Registration Statement on Form S-3 (the 2016 Shelf Registration Statement) with the SEC to sell up to approximately $33.8 million of common stock. The 2016 Shelf Registration Statement (file No. 333-215315) was declared effective by the SEC on January 9, 2017. No sales have been conducted to date under the 2016 Shelf Registration Statement.

2017 Shelf Registration Statement

On July 12, 2017, the Company filed a universal shelf Registration Statement on Form S-3 (the 2017 Shelf Registration Statement) with the SEC to sell up to $150 million of common stock, preferred stock, warrants to purchase common stock, or debt securities (including debt securities that may be convertible or exchangeable for common stock or other securities), which securities may be offered separately or together in units or multiple series. The 2017 Shelf Registration Statement (file No. 333-219259) was declared effective by the SEC on July 26, 2017. The Company has to date conducted the following sales of its securities under the 2017 Shelf Registration Statement:

(a) On November 28, 2017, the Company filed a prospectus supplement and offered for sale 16,428,572 shares of its common stock at a price of $3.50 per share in an underwritten public offering. The Company received gross proceeds in the public offering of approximately $57.5 million before underwriting commission and incurred expenses of approximately $3.7 million.

As of June 30, 2018, there is approximately $92.5 million available for future sale under the 2017 Shelf Registration Statement.

11. Stock Compensation.

Stock Options

During the three and six-month periods ended June 30, 2018, the Company granted seven-year options to purchase an aggregate of 945,000 and 2,717,500 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling $776,510 and $1,747,850 respectively, during the three and six-month periods ended June 30, 2018. During the three and six-month periods ended June 30, 2018, respectively, 565,000 and 1,309,998 options vested.

During the three and six-month periods ended June 30, 2017, the Company granted seven-year options to purchase an aggregate of 15,000 and 1,535,000 shares, respectively, of the Company's common stock to employees and directors. The Company recorded stock-based compensation related to stock options totaling $619,754 and $1,355,290 respectively, during the three and six-month periods ended June 30, 2017. During the three and six-month periods ended June 30, 2017, respectively, 621,667 and 876,667 options vested.

During the three and six-month periods ended June 30, 2018, options to purchase 10,000 shares and 46,666 shares, respectively, of the Company's common stock were exercised, with proceeds of $8,500 and $41,532, respectively, to the Company. No options were exercised during the three and six-months periods ended in June 30, 2017.

As of June 30, 2018, there was approximately $6,130,000 of unrecognized compensation expense related to non-vested stock option awards granted under the 2014 and 2018 Stock Incentive Plans. The cost is expected to be recognized over a weighted average period of approximately 2.45 years.

Restricted Stock Units

No restricted stock units were granted during the three and six-month periods ended June 30, 2018 and 2017. No stock-based compensation related to restricted stocks was recorded during the three and six-months periods ended June 30, 2018. The Company recorded stock-based compensation related to restricted stock units totaling $18,815 and $37,423, respectively, during the three and six-month periods ended June 30, 2017. All restricted stock units were vested as of December 31, 2017.

Common Stock

During both the three and six-month periods ended June 30, 2018, the Company granted 3,094 net shares of common stock to employees as compensation. The Company recorded stock-based compensation related to common stock issued to employees totaling approximately $15,000 during both the three and six-month periods ended June 30, 2018. There were no grants of common stock to employees during the three and six-months periods ended June 30, 2017.

12. Subsequent Events.

Subsequent to quarter end, effective July 26, 2018, the Company settled a dispute with BioMarin and the former Huxley stockholders regarding the milestone payments due to the former Huxley stockholders if certain milestones are achieved by the Company. See Note 8.

ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

Introduction

Management's Discussion and Analysis of Financial Condition and Results of Operations (MD&A) is intended to provide an understanding of our financial condition, changes in financial condition and results of operations. The discussion and analysis is organized as follows:

- *Overview.* This section provides a general description of our business and information about the current status of our business that we believe is important in understanding our financial condition and results of operations.
- *Basis of Presentation.* This section provides information about key accounting estimates and policies that we followed in preparing our consolidated financial statements for the second quarter and first half of fiscal 2018.
- *Critical Accounting Policies and Estimates*. This section discusses those accounting policies that are both considered important to our financial condition and results of operations, and require significant judgment and estimates on the part of management in their application. All of our significant accounting policies, including our critical accounting policies, are also summarized in the notes to our interim consolidated financial statements that are included in this report.
- *Results of Operations*. This section provides an analysis of our results of operations for the three and six-months ended June 30, 2018 as compared to the same periods ended June 30, 2017.
- *Liquidity and Capital Resources*. This section provides an analysis of our cash flows, capital resources, off-balance sheet arrangements and our outstanding commitments, if any.
- *Caution Concerning Forward-Looking Statements*. This section discusses how certain forward-looking statements made throughout this MD&A and in other sections of this report are based on management's present expectations about future events and are inherently susceptible to uncertainty and changes in circumstance.

Overview

We are a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare, debilitating, chronic neuromuscular and neurological diseases. We currently have three drug candidates in development:

- Firdapse®

  In October 2012, we licensed the North American rights to Firdapse®, a proprietary form of amifampridine phosphate, or chemically known as 3,4-diaminopyridine phosphate, from BioMarin Pharmaceutical Inc. (BioMarin). In August 2013, we were granted "breakthrough therapy designation" by the U.S. Food & Drug Administration (FDA) for Firdapse® for the treatment of patients with Lambert-Eaton Myasthenic Syndrome, or LEMS, a rare and sometimes fatal autoimmune disease characterized by muscle weakness. Further, the FDA has granted Orphan Drug Designation for Firdapse® for the treatment of patients with LEMS, Congenital Myasthenic Syndromes, or CMS and Myasthenia Gravis (MG).

  The chemical entity, amifampridine (3,4-diaminopyridine, or 3,4-DAP), has never been approved by the FDA for any indication. Because amifampridine phosphate (Firdapse®) has been granted Orphan Drug designation for the treatment of LEMS, CMS and MG by the FDA, the product is eligible to receive seven years of marketing exclusivity for either or all of these indications. Further, if we are the first pharmaceutical company to obtain approval for an amifampridine product, of which there can be no assurance, we will also be eligible to receive five years of marketing exclusivity with respect to the use of this product for any indication, running concurrently with the seven years of orphan marketing exclusivity described above (if both exclusivities are granted).

We previously sponsored two multi-center, randomized, placebo-controlled Phase 3 trials evaluating Firdapse® for the treatment of LEMS, both of which were successful. On March 29, 2018, we reported that we had submitted an NDA to the FDA for Firdapse® for the symptomatic treatment of LEMS, and on May 29, 2018, we reported FDA acceptance of our NDA and Priority Review Status for Firdapse® for the symptomatic treatment of LEMS, with a Prescription Drug User Fee Act (PDUFA) goal date of November 28, 2018. There can be no assurance that our NDA for Firdapse® for LEMS will be approved by the FDA or the timing of any such approval.

We are currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of certain types of CMS. This trial, which will include approximately 23 adult and pediatric subjects, is being conducted at trial sites around the United States and Canada. We are also currently working to add one or more additional sites outside the United States. Details of this trial are available on www.clinicaltrials.gov (NCT02562066). Based on currently available information, we currently expect to report top-line results from this trial in the first half of 2019. There can be no assurance that any trial we conduct evaluating Firdapse® for the treatment of CMS will be successful or whether any NDA or NDA supplement that we may submit for Firdapse® for the treatment of CMS in the future will be filed by the FDA for review and approved.

We are also currently conducting a Phase 3 clinical trial evaluating Firdapse® for the treatment of MuSK antibody positive myasthenia gravis (MuSK-MG). The trial is a multi-site, international (U.S. and Italy), double-blind, placebo-controlled, clinical trial that is targeted to enroll approximately 60 subjects diagnosed with MuSK-MG. The trial will also enroll up to 10 generalized myasthenia gravis patients who will be assessed with the same clinical endpoints but achieving statistical significance in this subgroup of patients is not required and only summary statistics will be provided. We initiated this trial in January 2018 and are currently enrolling subjects. We currently expect to report top-line results from this trial in the second half of 2019. Details of this trial are available on www.clinicaltrials.gov (NCT03304054).

Finally, we have begun conducting a Phase 3 clinical study evaluating Firdapse® as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3. The study is designed as a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study to evaluate the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. The study is planned to include approximately 12 patients, and we currently expect to report top-line results from the study in the second half of 2019.

There can be no assurance that our currently ongoing trials evaluating Firdapse® for the treatment of CMS, MuSK-MG or SMA Type 3, or any trials we may undertake in the future to evaluate Firdapse® for the treatment of other rare, similar neuromuscular diseases, will be successful. Further, there can also be no assurance that the FDA will ever approve Firdapse® for any of these indications.

Now that our NDA for Firdapse® for the treatment of LEMS has been accepted for filing by the FDA, we are substantially increasing our activities to prepare for the potential marketing of Firdapse® in the United States as early as the beginning of 2019. During June 2018, we announced the appointment of Daniel J. Brennan as Chief Commercial Officer, and we are currently building our sales and marketing team to prepare for the launch of Firdapse® (assuming we obtain approval to commercialize the product), which will include our marketing and sales team, our patient education and support programs and our market access/reimbursement operations. We currently expect to launch and sell Firdapse® through a field force of approximately 15-20 employees experienced in rare diseases. At launch, the sales force will focus on the approximately 900 neuromuscular specialists who we believe most often treat neuromuscular diseases such as LEMS. We also expect to continue to work with several rare disease advocacy organizations to help increase awareness of LEMS and CMS and to provide education for the physicians who treat these rare diseases and the patients they treat, and we expect to have a field-based force of 4-6 medical science liaisons who will help educate the medical communities and patients about these illnesses.

We currently intend to use a specialty pharmacy model to provide reimbursement, clinical and distribution support for Firdapse® and to develop cost-sharing and patient assistance programs to support qualified, commercially insured patients, federal- and state-insured patients, and uninsured or under-insured patients. We may also donate money to independent charitable foundations dedicated to this cause. Our ultimate goal is to ensure that no LEMS patient is denied access to Firdapse® for financial reasons.

- CPP-115

  We are developing CPP-115, a GABA aminotransferase inhibitor that, based on our preclinical studies to date, we believe is a more potent form of vigabatrin, and may have fewer side effects (e.g., visual field defects) than those associated with vigabatrin. We are hoping to develop CPP-115 for the treatment of refractory infantile spasms and possibly for the treatment of adult refractory patients with Tourette's Disorder. CPP-115 has been granted Orphan Drug designation by the FDA for the treatment of infantile spasms and Orphan Medicinal Product designation in the European Union, or E.U., for West syndrome (a form of infantile spasms).

  Recently, we became aware that certain patents granted to Northwestern to a new GABA aminotransferase inhibitor and derivative of vigabatrin were derived from CPP-115. As such, it is our position that Northwestern is currently in breach of the license agreement based on its failure to transfer these new patent rights to us. Discussions to resolve this dispute are ongoing. There can be no assurance as to the outcome of this matter, and, if the matter is not amicably resolved, we intend to pursue our rights under the license agreement.

- Generic Sabril®

  In September 2015, we announced the initiation of a project to develop generic versions of Sabril® (vigabatrin) in two dosage forms: tablets and powder sachets. Sabril® is marketed by Lundbeck Inc. in the United States in both dosage forms for the treatment of infantile spasms and refractory complex partial seizures. There can be no assurance that we will be successful in these efforts or that any abbreviated new drug applications (ANDAs) that we submit for vigabatrin will be accepted for review or approved.

  We are also continuing our efforts to seek a partner to work with us in furthering the development of generic Sabril®. However, no agreements have been entered into to date.

  There can be no assurance that we will ever successfully commercialize a generic version of Sabril®.

Available Capital Resources

Based on forecasts of available cash, we believe that we have sufficient resources to support our currently anticipated operations through 2019 (without considering revenues and cash receipts that may be received in 2019 if we are successful in obtaining an approval for Firdapse® and launching the product in 2019, of which there can be no assurance. There can be no assurance that we will ever be in a position to commercialize any of our drug candidates or that we will obtain any additional funding that we may require in the future. See "Liquidity and Capital Resources" below for further information on our liquidity and cash flow.

Basis of presentation

Revenues.

We are a development stage company and have had no revenues from product sales to date. We will not have revenues from product sales until such time as we receive approval of our drug candidates, successfully commercialize our products or enter into a licensing agreement which may include up-front licensing fees, of which there can be no assurance.

Research and Development Expenses.

Our research and development expenses consist of costs incurred for company-sponsored research and development activities, as well as support for selected investigator-sponsored research. The major components of research and development costs include preclinical study costs, clinical manufacturing costs, clinical study and trial expenses, insurance coverage for clinical trials, consulting, scientific advisors and other third-party costs, salaries and employee benefits, stock-based compensation expense, supplies and materials and allocations of various overhead costs related to our product development efforts. To date, all of our research and development resources have been devoted to the development of CPP-109 (our version of vigabatrin), CPP-115, and Firdapse®, and we expect this to continue for the foreseeable future.

Our cost accruals for clinical studies and trials are based on estimates of the services received and efforts expended pursuant to contracts with numerous clinical study and trial sites and clinical research organizations (CROs). In the normal course of our business we contract with third parties to perform various clinical study and trial activities in the on-going development of potential products. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Payments under the contracts depend on factors such as the achievement of certain events or milestones, the successful enrollment of patients, the allocation of responsibilities among the parties to the agreement, and the completion of portions of the clinical study or trial or similar conditions. The objective of our accrual policy is to match the recording of expenses in our consolidated financial statements to the actual services received and efforts expended. As such, expense accruals related to preclinical and clinical studies or trials are recognized based on our estimate of the degree of completion of the event or events specified in the specific study or trial contract. We monitor service provider activities to the extent possible; however, if we underestimate activity levels associated with various studies or trials at a given point in time, we could be required to record significant additional research and development expenses in future periods. Preclinical and clinical study and trial activities require significant up-front expenditures. We anticipate paying significant portions of a study or trial's cost before such study or trial begins, and incurring additional expenditures as the study or trial progresses and reaches certain milestones.

Selling and Marketing Expenses.

We do not currently have any selling or marketing expenses. We are currently incurring substantial costs to build our commercial team for a potential launch of Firdapse® in the first quarter of 2019. Such pre-commercialization costs are included in general and administrative expenses.

General and Administrative Expenses.

Our general and administrative expenses consist primarily of salaries and personnel expenses for accounting, corporate, compliance and administrative functions. Other costs include administrative facility costs, regulatory fees, insurance, pre-commercialization costs, and professional fees for legal, information technology, accounting and consulting services.

Stock-Based Compensation.

We recognize expense for the fair value of all stock-based awards to employees, directors, and consultants in accordance with U.S. GAAP. For stock options, we use the Black-Scholes option valuation model in calculating the fair value of the awards.

Warrants Liability.

We issued warrants to purchase shares of our common stock as part of an equity financing that we completed in October 2011. In accordance with U.S. GAAP, we recorded the fair value of those warrants as a liability in the consolidated balance sheet using a Black-Scholes option-pricing model. We remeasured the fair value of this warrants liability at each reporting date until the warrants were exercised or until the unexercised warrants expired on May 2, 2017. Changes in the fair value of the warrants liability was reported in the consolidated statements of operations as income or expense. The fair value of the warrants liability was subject to significant fluctuation over the life of these warrants based on changes in the inputs to the Black-Scholes option-pricing model, including our common stock price, expected volatility, expected term, the risk-free interest rate and dividend yield.

Income Taxes.

We have incurred operating losses since inception. Our net deferred tax asset has a 100% valuation allowance as of June 30, 2018 and December 31, 2017, as we believe it is more likely than not that the deferred tax asset will not be realized. If an ownership change, as defined under Internal Revenue Code Section 382, occurs, the use of any of our carry-forward tax losses may be subject to limitation.

As required by ASC 740, *Income Taxes*, we would recognize the financial statement benefit of a tax position only after determining that the relevant tax authority would more likely than not sustain the position following an audit. For tax positions meeting the more-likely-than-not threshold, the amount recognized in the financial statements is the largest benefit that has a greater than 50 percent likelihood of being realized upon ultimate settlement with the relevant tax authority.

Recently Issued Accounting Standards.

For discussion of recently issued accounting standards, please see Note 2, “Basis of Presentation and Significant Accounting Policies,” in the interim consolidated financial statements included in this report.

Non-GAAP Financial Measures.

We prepare our consolidated financial statements and footnotes thereto which accompany this report in accordance with U.S. GAAP (GAAP). To supplement our financial results presented on a GAAP basis, we may use non-GAAP financial measures in our reports filed with the Commission and/or in our communications with investors. Non-GAAP measures are provided as additional information and not as an alternative to our consolidated financial statements presented in accordance with GAAP. Our non-GAAP financial measures are intended to enhance an overall understanding of our current financial performance. We believe that the non-GAAP financial measures that we present provide investors and prospective investors with an alternative method for assessing our operating results in a manner that we believe is focused on the performance of ongoing operations and provide a more consistent basis for comparison between periods.

The non-GAAP financial measure that we have historically presented excludes from the calculation of net loss the expense (or the income) associated with the change in fair value of the liability-classified warrants. Further, we have historically reported non-GAAP net loss per share, which is calculated by dividing non-GAAP net loss by the weighted average common shares outstanding.

Any non-GAAP financial measures that we report should not be considered in isolation or as a substitute for comparable GAAP accounting, and investors should read them in conjunction with our consolidated financial statements and notes thereto prepared in accordance with GAAP. Finally, the non-GAAP measures of net loss that we may use may be different from, and not directly comparable to, similarly titled measures used by other companies.

Critical Accounting Policies and Estimates

Our discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these consolidated financial statements requires us to make judgments, estimates, and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses and disclosures of contingent assets and liabilities. For a full discussion of our accounting policies, please refer to Note 2 on the Financial Statements included in our 2017 Annual Report on Form 10-K filed with the SEC. Our most critical accounting policies and estimates include: accounting for research and development expenses and stock-based compensation, measurement of fair value, fair value of warrants liability, income taxes and reserves. We continually evaluate our judgments, estimates and assumptions. We base our estimates on the terms of underlying agreements, our expected course of development, historical experience and other factors that we believe are reasonable based on the circumstances, the results of which form our management’s basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates. There have been no material changes to our critical accounting policies and estimates from the information provided in Part II, Item 7, *Management’s Discussion and Analysis of Financial Condition and Results of Operations* included in our 2017 Annual Report on Form 10-K.

Results of Operations

Revenues.

We had no revenues for the three and six-month periods ended June 30, 2018 and 2017.

Research and Development Expenses.

Research and development expenses for the three and six-month periods ended June 30, 2018 were $3,704,824 and $6,963,866, respectively, including stock-based compensation expense in each of the three and six-month periods of $285,625 and $579,940, respectively. Research and development expenses for the three and six-month periods ended June 30, 2017 were $2,451,751 and $5,265,680 respectively, including stock-based compensation expense in each of the three and six-month periods of $223,552 and $429,904, respectively. Research and development expenses, in the aggregate, represented approximately 58% and 57% of total operating costs and expenses for the three and six-month periods ended June 30, 2018 and 59% and 59% for the three and six-month periods ended June 30, 2017, respectively. The stock-based compensation is non-cash and relates to the expense of stock options awards and other non-cash stock compensation to certain employees.

Expenses for research and development for the six months ended June 30, 2018, excluding stock-based compensation, increased compared to amounts expended in the same period in 2017. The increase in research and development expenses for the six months ended June 30, 2018 when compared to the same period in 2017 is primarily due to increases in consulting expenses as we prepared to submit our NDA for Firdapse® during the first quarter of 2018, milestone expenses in connection with the acceptance of our NDA submission in May 2018, expenses from our medical affairs program, and compensation and related personnel costs as we expand our headcount to support our currently ongoing trials and programs. We expect that research and development costs will continue to be substantial during the balance 2018 as we work towards completing trials evaluating Firdapse® for the treatment of CMS, MuSK-MG and SMA Type 3, continue our Expanded Access Program for Firdapse® and our other development programs, and prosecute our recently accepted NDA submission for Firdapse® for LEMS.

Selling and Marketing Expenses.

We had no selling expenses for the six-month periods ended June 30, 2018 and 2017.

During the fourth quarter of 2017, as we moved closer to submitting an NDA for Firdapse®, we re-started the development of our commercialization plans for Firdapse® and following the acceptance of our NDA for Firdapse® for the treatment of LEMS, we accelerated our efforts to build our commercial team for a potential launch of Firdapse® in the first quarter of 2019. Pre-commercialization costs are included in general and administrative expenses.

General and Administrative Expenses.

General and administrative expenses for the three and six months ended June 30, 2018 were $2,631,031 and $5,305,429, respectively, including stock-based compensation expense in each of the three and six-month periods ending June 30, 2018 of $505,882 and $1,182,907, respectively. General and administrative expenses for the three and six months ended June 30, 2017 were $1,729,520 and $3,595,462, respectively, including stock-based compensation expense in each of the three and six-month periods ending June 30, 2017 of $415,017 and $962,809, respectively. General and administrative expenses represented 42% and 43% of total operating costs and expenses for the three and six months ended June 30, 2018 and 41% and 41% for the three and six months ended June 30, 2017, respectively. The increase in general and administrative expenses for the six months ended June 30, 2018 when compared to the same period in 2017 is primarily due to increases in pre-commercialization expenses, headcount and corporate expenses as we build up our infrastructure and commercial programs in preparation for a potential Firdapse® launch in 2019. We expect that general and administrative costs, including pre-commercialization costs, will continue to increase in 2018 compared with the general and administrative costs incurred in 2017, as we continue to expand our operations in preparation for a potential launch of Firdapse® in 2019.

As discussed above, pre-commercialization expenses are included in general and administrative expenses, and amounted to $832,208 and $1,463,199 respectively, for the three and six-month periods ended June 30, 2018 and $241,084 and $407,289 respectively, for the three and six-month periods ended June 30, 2017.

Stock-Based Compensation.

Total stock-based compensation for the three and six-month periods ended June 30, 2018 were $791,507 and $1,762,847 and for the three and six-month periods ended June 30, 2017 were $638,569 and $1,392,713, respectively. The increase in stock-based compensation for the six-month periods ended June 30, 2018, when compared to the same period in 2017, is primarily due to the expense of options granted to employees and directors during the first quarter of 2018 in connection with 2017 year-end grants and the effect of grants to new employees as we increase our headcount to build up our infrastructure and commercial programs in preparation for a potential launch of Firdapse® in 2019.

Change in Fair Value of Warrants Liability.

In connection with our October 2011 equity offering, we issued warrants to purchase an aggregate of 1,523,370 shares of common stock. As of May 2, 2017, all of the 2011 warrants were either exercised or had expired. During the period that the 2011 warrants were outstanding, the fair value of the warrants liability was determined at the end of each reporting period with the resulting gains or losses recorded as the change in fair value of warrants liability in the consolidated statements of operations.

No gain or loss was recognized for the three and six months ended June 30, 2018, as all 2011 warrants that were not exercised expired on May 2, 2017. For the three and six months ended June 30, 2017, we recognized a gain of $210,331 and a loss of $186,904, respectively, due to the change in the fair value of the warrants liability. The gain during the three months ended June 30, 2017 was principally a result of the decrease of our stock price between March 31, 2017 and the warrants liability expiration date on May 2, 2017. The loss during the six months ended June 30, 2017 was principally a result of the increase of our stock price between December 31, 2016 and the warrants liability expiration date on May 2, 2017.

Other Income, Net.

We reported other income, net in all periods relating to our investment of funds received from offerings of our securities. The increase in other income, net for the six months ended June 30, 2018 when compared to the same period in 2017 is primarily due to higher yields on investments and higher invested balances. Other income, net, consists of interest income, dividend income, unrealized and realized gain (loss) on trading securities and realized gain (loss) on available-for-sale securities, if any. These proceeds are used to fund our drug development activities and our operations. Substantially all such funds were invested in short-term interest-bearing obligations and short-term bond funds.

Income Taxes.

We have incurred net operating losses since inception. For the three and six-month periods ended June 30, 2018 and 2017 we have applied a 100% valuation allowance against our deferred tax asset as we currently believe that it is more likely than not that the deferred tax asset will not be realized.

Net Loss.

Our net loss was $5,965,140 and $11,665,032, respectively, for the three and six months ended June 30, 2018 ($0.06 and $0.11, respectively, per basic and diluted share) as compared to a net loss of $3,879,901 and $8,847,030, respectively, for the three and six months ended June 30, 2017 ($0.05 and $0.11, respectively, per basic and diluted share).

Non-GAAP Net Loss.

Our non-GAAP net loss for the three and six months ended June 30, 2018 was the same as our GAAP net loss, as there were no non-GAAP adjustments. Our non-GAAP net loss, which excludes for the three and six months ended June 30, 2017 a gain of $210,331 and a loss of $186,904, respectively, associated with the change in the fair value of liability classified warrants, was $4,090,232 and $8,660,126, respectively, for the three and six months ended June 30, 2017 ($0.05 and $0.10, respectively, per basic and diluted share).

Liquidity and Capital Resources

Since our inception, we have financed our operations primarily through equity issuances, government grants, and an investment by a strategic purchaser. At June 30, 2018, we had cash and investments aggregating $73.4 million and working capital of $66.0 million. At December 31, 2017, we had cash and investments aggregating $84.0 million and working capital of $80.9 million. At June 30, 2018, substantially all of our cash and cash equivalents were deposited with one financial institution, and such balances were in excess of federally insured limits.

We have to date incurred operating losses, and we expect these losses to be substantial in the future as we continue our drug development programs and prepare for the commercialization of our drug candidates. We anticipate using current cash on hand to finance these activities. It will likely be some time before we obtain the necessary regulatory approvals to commercialize one or more of our product candidates in the United States.

Based on forecasts of available cash, we believe that we have sufficient resources to support our currently anticipated operations through 2019 (without considering revenues and cash receipts that may be received in 2019 if we are successful in obtaining an approval for Firdapse® and launching the product in 2019, of which there can be no assurance). There can be no assurance that we will ever be in a position to commercialize any of our drug candidates or that we will obtain any additional funding that we may require in the future.

At the present time, we will require additional funding for future studies or trials, other than those described as being on-going in this report. We may also require additional working capital to support our operations beyond that time, depending on when and if we are able to launch Firdapse® and whether the results are cash flow positive. There can be no assurance as to the amount of any such funding that will be required for these purposes or whether any such funding will be available to us when it is required.

In that regard, our future funding requirements will depend on many factors, including:

- the scope, rate of progress and cost of our clinical trials and other product development activities;
- future clinical trial results;
- the terms and timing of any collaborative, licensing and other arrangements that we may establish;
- the cost and timing of regulatory approvals;
- the cost and delays in product development as a result of any changes in regulatory oversight applicable to our products;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the effect of competition and market developments;
- the cost of filing and potentially prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the extent to which we acquire or invest in other products.

We plan to raise additional funds that we may require in the future, through public or private equity offerings, debt financings, corporate collaborations or other means. We also may seek governmental grants for a portion of the required funding for our clinical trials and preclinical trials. We may also seek to raise capital to fund additional product development efforts or product acquisitions, even if we have sufficient funds for our planned operations. Any sale by us of additional equity or convertible debt securities could result in dilution to our stockholders. There can be no assurance that any such required additional funding will be available to us at all or available on terms acceptable to us. Further, to the extent that we raise additional funds through collaborative arrangements, it may be necessary to relinquish some rights to our technologies or grant sublicenses on terms that are not favorable to us. If we are not able to secure additional funding when needed, we may have to delay, reduce the scope of or eliminate one or more research and development programs, which could have an adverse effect on our business.

On July 12, 2017, we filed a shelf registration statement with the SEC to sell up to $150,000,000 of common stock, preferred stock, warrants to purchase common stock, debt securities and units consisting of one or more of such securities (the “2017 Shelf Registration Statement”). The 2017 Shelf Registration Statement (file no. 333-219259) was declared effective by the SEC on July 26, 2017. We have completed one offering under the 2017 Shelf Registration Statement:

- On November 28, 2017, we raised net proceeds of approximately $53.8 million from the sale of 16,428,572 shares of our common stock.

On December 23, 2016, we filed a shelf registration statement with the SEC to sell up to $33,842,512 of common stock (the “2016 Shelf Registration Statement”). This shelf registration statement was declared effective by the SEC on January 9, 2017. We have made no sales under the 2016 Shelf Registration Statement.

At June 30, 2018, the full amount of our 2016 Shelf Registration Statement and $92,499,998 of our 2017 Shelf Registration Statement remains available for future sales. However, if our public float (the market value of our common stock held by non-affiliate stockholders) were to fall below $75 million, we would be subject to a further limitation under which we could sell no more than one-third (1/3) of our public float during any 12-month period. Further, the number of shares that we can sell at any one time may be limited under certain circumstances to 20% of the outstanding common stock under applicable NASDAQ marketplace rules.

Cash Flows.

Net cash used in operating activities was $10,587,846 and $7,079,864, respectively, for the six-month periods ended June 30, 2018 and 2017. During the six months ended June 30, 2018, net cash used in operating activities was primarily attributable to our net loss of $11,665,032, and decreases of $1,022,982 in accounts payable, and $175,273 in accrued expenses and other liabilities. This was partially offset by a $496,403 decrease in prepaid expenses and other current assets and deposits, and $1,779,038 of other non-cash expenses. During the six months ended June 30, 2017, net cash used in operating activities was primarily attributable to our net loss of $8,847,030, decreases of $240,831 in accounts payable and $23,921 in accrued expenses and other liabilities. This was partially offset by a $426,386 decrease in prepaid expenses and other current assets and deposits, $186,904 of non-cash change in fair value of warrants liability and $1,418,628 of other non-cash expenses. Such additional non-cash expenses consist of depreciation and stock-based compensation expense.

Net cash used in investing activities was $37,058,001 and $34,910, respectively, for the six-month periods ended June 30, 2018 and June 30, 2017, consisting primarily of purchases of investments.

Net cash provided by financing activities during the six-month period ended June 30, 2018 was $37,084, consisting primarily of proceeds from the exercise of options to purchase common stock. Net cash provided by financing activities during the six-month period ended June 30, 2017 was $1,805,437, consisting of the net proceeds from exercise of warrants.

Contractual Obligations.

We have entered into the following contractual arrangements:

- *Payments to BioMarin and others under our license agreement with BioMarin.* We have agreed to pay certain payments under our license agreement with BioMarin.

    - *Royalties:* We have agreed to pay (i) royalties to BioMarin for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in the license agreement) in North America for any calendar year for sales up to $100 million, and 10% of net sales in North America in any calendar year in excess of $100 million; and (ii) royalties to the third-party licensor of the rights sublicensed to us for seven years from the first commercial sale of Firdapse® equal to 7% of net sales (as defined in the license agreement between BioMarin and the third-party licensor) in any calendar year.

      Milestone Payments: Additionally, our license agreement with BioMarin requires that we pay certain milestone payments that BioMarin is obligated to pay to both a third-party licensor of the rights that have been sublicensed to us and to the former stockholders of Huxley Pharmaceuticals (“Huxley”) under an earlier stock purchase agreement between BioMarin and the former Huxley stockholders.

      With respect to the third party licensor of the rights that have been sublicensed to us, we have agreed to pay: (i) $150,000 upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS (which amount was paid during the second quarter of 2018 after acceptance by the FDA of the Company’s NDA for Firdapse® for LEMS), and (ii) approximately $3.0 million of which will be due upon the upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (which milestone payment has not yet been earned).

With respect to the former Huxley stockholders, we had agreed that we would pay the following milestone payments if either of the following milestones were satisfied before April 20, 2018: (i) $2,425,000 upon acceptance by the FDA of a filing of an NDA for Firdapse® for the treatment of LEMS, and (ii) approximately $4,200,000 of which was to be paid upon the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS. Since neither of these milestones were met on or before April 20, 2018, these milestone payments were never earned. However, prior to April 20, 2018, we were advised that the former Huxley stockholders intended to take legal action against BioMarin and us seeking payment of the milestone payments due to them even if the milestones were achieved after their expiration date (April 20, 2018). While we disputed our obligation to pay either of the above described milestone payments if these milestones were achieved after April 20, 2018, in an agreement which was executed and became effective on July 26, 2018, we, BioMarin and the former Huxley stockholders agreed to amicably resolve this matter. As part of the settlement, and without admitting any liability, we agreed to pay the former Huxley stockholders two new milestone payments in lieu of the milestone payments described above: (i) a $1.0 million milestone payment due upon execution of the settlement agreement, and (ii) a $1.0 million payment due on the unconditional approval by the FDA of an NDA for Firdapse® for the treatment of LEMS (which milestone payment has not yet been earned). Further, as part of the settlement agreement, we, BioMarin and the former Huxley stockholders entered into mutual releases regarding these matters.

- *Cost Sharing Payments.* We have agreed to share in the cost of certain post-marketing studies conducted by BioMarin, and, as of June 30, 2018, we had paid BioMarin $3.8 million related to expenses in connection with Firdapse® studies and trials.

- *Payments to Northwestern University under our license agreement*. Under our license agreement with Northwestern, from which we derive our rights to CPP-115, we have paid to date $424,885, had accrued liabilities of $305,000, at June 30, 2018 in the accompanying consolidated balance sheet, and owe certain milestone payments in future years if we do not cancel the license agreement. The next milestone payment of $300,000 is due on the earlier of successful completion of the first Phase 3 clinical trial of CPP-115 or August 27, 2018.

  Recently, we became aware that certain patents granted to Northwestern to a new GABA aminotransferase inhibitor and derivative of vigabatrin were derived from CPP-115. As such, it is our position that Northwestern is currently in breach of the license agreement based on its failure to transfer these new patent rights to us. Discussions to resolve this dispute are ongoing. There can be no assurance as to the outcome of this matter, and, if the matter is not amicably resolved, we intend to pursue our rights under the license agreement.

- *Employment agreements.* We have entered into an employment agreement with our Chief Executive Officer that requires us to make base salary payments of approximately $525,000 in 2018. The agreement expires in November 2020.

- *Lease for office space.* We operate our business in leased office space in Coral Gables, Florida. We currently lease approximately 5,200 square feet of office space for which we pay annual rent of approximately $200,000.

Off-Balance Sheet Arrangements.

We currently have no debt or capital leases. We have operating leases for our office facilities. We do not have any off-balance sheet arrangements as such term is defined in rules promulgated by the SEC.

Caution Concerning Forward-Looking Statements

This Current Report on Form 10-Q contains “forward-looking statements”, as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding our expectations, beliefs, plans or objectives for future operations and anticipated results of operations. For this purpose, any statements contained herein that are not statements of historical fact may be deemed to be forward-looking statements. Without limiting the foregoing, “believes”, “anticipates”, “proposes”, “plans”, “expects”, “intends”, “may”, and other similar expressions are intended to identify forward-looking statements. Such statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by such forward looking statements. The forward-looking statements made in this report are based on current expectations that involve numerous risks and uncertainties.

The successful development and commercialization of our current drug candidates is highly uncertain. We cannot reasonably estimate or know the nature, timing, or estimated expenses of the efforts necessary to complete the development of, or the period in which material net cash inflows are expected to commence due to the numerous risks and uncertainties associated with developing such products, including the uncertainty of:

- our estimates regarding anticipated capital requirements and our need for additional financing;
- the risk that another pharmaceutical company will receive an approval for its formulation of 3,4-DAP for the treatment of LEMS, CMS, or any other indication, before we do;
- whether Firdapse® will be determined to be safe and effective and approved for commercialization for any indication;
- whether the receipt of breakthrough therapy designation for Firdapse® for LEMS will affect the likelihood that the product will be found to be safe and effective;
- whether as part of the FDA review of any NDA that we may submit for filing for Firdapse®, the tradename Firdapse®, which is the tradename used for the same product in Europe, will be approved for use for the product in the United States;
- whether, assuming Firdapse® is approved for commercialization, we will be able to develop a sales and marketing organization that can successfully market Firdapse® while maintaining full compliance with applicable federal and state laws, rules and regulations;
- whether our estimates of the size of the market for our drug candidates will turn out to be accurate;
- the pricing of our products that we may be able to achieve if we are granted the ability to commercialize our drug candidates;
- assuming Firdapse® is approved for commercialization, the timing of third party payor coverage and reimbursement for the product;
- whether, even if Firdapse® is approved for commercialization, we will be successful in commercializing Firdapse®;
- changes in the healthcare industry and the effect of political pressure from President Trump and Congress to reduce prescription drug costs;
- changes to the healthcare industry occasioned by any future repeal and replacement of the Affordable Care Act, in laws relating to the pricing of drug products, or changes in the healthcare industry generally;
- the scope, rate of progress and expense of our clinical trials and studies, pre-clinical studies, proof-of-concept studies, and our other drug development activities, and whether our trials and studies will be successful;
- our ability to complete our trials and studies on a timely basis and within the budgets we establish for such trials and studies;
- whether the trials that we are currently undertaking to evaluate Firdapse® for the treatment of CMS, MuSK-MG and SMA Type 3 or any other trials that we undertake in the future will be successful;
- whether CPP-115 will be determined to be safe for humans;
- whether CPP-115 will be determined to be effective for the treatment of infantile spasms, or any other disorder;

- whether we can successfully design and complete bioequivalence studies of our versions of vigabatrin compared to Sabril® that are acceptable to the FDA;
- whether any ANDAs that we submit for a generic version of Sabril® will be accepted for filing and approved (and the timing of any such acceptances and approvals); and
- the ability of our third-party suppliers and contract manufacturers to maintain compliance with current Good Manufacturing Practices (cGMP).

Our current plans and objectives are based on assumptions relating to the development of our current drug candidates. Although we believe that our assumptions are reasonable, any of our assumptions could prove inaccurate. In light of the significant uncertainties inherent in the forward-looking statements we have made herein, which reflect our views only as of the date of this report, you should not place undue reliance upon such statements. We undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURE ABOUT MARKET RISK

Our market risks during the six months ended June 30, 2018 have not materially changed from those discussed in Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2017.

ITEM 4. CONTROLS AND PROCEDURES

a. We have carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of June 30, 2018, our disclosure controls and procedures were effective to ensure that the information required to be disclosed by us in the reports filed or submitted by us under the Exchange Act, was recorded, processed, summarized or reported within the time periods specified in the rules and regulations of the SEC, and include controls and procedures designed to ensure that information required to be disclosed by us in such reports was accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosures.

b. During the six months ended June 30, 2018, there were no changes in our internal controls or in other factors that could have a material effect, or are reasonably likely to have a material effect, on our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

The Company is not a party to any material legal proceedings.

ITEM 1A. RISK FACTORS

There are many factors that affect our business, our financial condition, and the results of our operations. In addition to the information set forth in this quarterly report, you should carefully read and consider “Item 1A. Risk Factors” in Part I, and “Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations” in Part II, of our 2017 Annual Report on Form 10-K filed with the SEC, which contain a description of significant factors that might cause our actual results of operations in future periods to differ materially from those currently expected or desired.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURE

Not applicable.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

10.1	Settlement Agreement, effective as of July 26, 2018, among the Company, BioMarin Pharmaceutical Inc. and Aceras BioMedical LLC, in its capacity as stockholder representative for the former stockholders of Huxley Pharmaceuticals, Inc.
31.1	Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer under Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation Linkbase
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Label Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase

SIGNATURES

Pursuant to the Securities Exchange Act of 1934, the Registrant has caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Catalyst Pharmaceuticals, Inc.

By: /s/ Alicia Grande
Alicia Grande
Vice President, Treasurer and Chief Financial Officer

Date: August 7, 2018

Exhibit 10.1

SETTLEMENT AGREEMENT

THIS SETTLEMENT AGREEMENT ("**Agreement**") is effective as of July 26, 2018 (the "**Effective Date**") by and among, (i) Aceras BioMedical LLC, a Delaware limited liability company ("**Aceras**"), in its capacity as Stockholder Representative for the former stockholders (the "**Stockholders**") of Huxley Pharmaceuticals, Inc., a Delaware corporation ("**Huxley**"), that are a party to the SPA (as such term is defined below), (ii) BioMarin Pharmaceutical Inc., a Delaware corporation ("**BioMarin**"), and (iii) Catalyst Pharmaceuticals, Inc. (f/k/a Catalyst Pharmaceutical Partners, Inc.), a Delaware corporation ("**Catalyst**"). Aceras, BioMarin, and Catalyst may be individually referred to herein as a "**Party**" and collectively referred to herein as the "**Parties**".

RECITALS

WHEREAS, pursuant to that certain Stock Purchase Agreement, dated as of October 20, 2009, by and among BioMarin, Huxley, and the Stockholders (the "**Original SPA**"), as amended by the First Amendment to Stock Purchase Agreement, dated March 26, 2010 ("**SPA Amendment 1**"), and the Second Amendment to Stock Purchase Agreement, dated October 26, 2012 ("**SPA Amendment 2**"), (the Original SPA, as amended by SPA Amendment 1 and SPA Amendment 2, the "**SPA**"), the Stockholders sold, assigned, transferred and delivered to BioMarin, and BioMarin purchased and acquired from the Stockholders, all right, title and interest in and to all of the issued and outstanding shares of capital stock of Huxley;

WHEREAS, pursuant to Section 1.4 of the SPA, BioMarin was obligated to make contingent payments (the "**SPA Milestone Payments**") to the Stockholders if certain regulatory and commercial milestones (the "**SPA Milestones**") were met before April 20, 2018 (the "**SPA Milestones Expiration Date**");

WHEREAS, pursuant to that certain License Agreement, dated as of October 26, 2012, as amended April 8, 2014, by and between BioMarin and Catalyst (the "**License Agreement**"), BioMarin licensed to Catalyst certain technology and granted to Catalyst certain rights previously acquired by BioMarin as part of its acquisition of Huxley under the SPA;

WHEREAS, pursuant to Sections 3.3, 6.2, and 7.5 of the License Agreement and Exhibit F thereto, Catalyst is obligated to satisfy BioMarin's obligations or allow BioMarin to satisfy its obligations to the Stockholders under the SPA that are related to the subject matter of the License Agreement, including without limitation, make the SPA Milestone Payments if they are required to be made under the terms of the SPA;

WHEREAS, the SPA Milestones described in Sections 1.4(g) and 1.4(h) of the SPA did not occur before the SPA Milestones Expiration Date, and therefore, the related SPA Milestone Payments were not payable and have expired by their terms;

WHEREAS, disputes arose among the Parties regarding the Parties' respective obligations related to the SPA Milestones and SPA Milestone Payments described in Sections 1.4(g) and 1.4(h) of the SPA, and, in order to fully resolve such disputes, the Parties have entered into this Agreement; and

WHEREAS, Aceras and Catalyst desire to establish two new regulatory milestones, as further described below in this Agreement.

NOW, THEREFORE, in consideration of the foregoing, the mutual promises and covenants contained in this Agreement, and for other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties agree as follows:

1. RELATION TO OTHER AGREEMENTS

1.1. This Agreement does not amend either the SPA or the License Agreement.

1.2. The SPA continues in full force and effect in accordance with its terms and continues to be an agreement between BioMarin and the Stockholders. The rights granted to Catalyst under Section 5.11(b) (Noncompetition) of the SPA remain in full force and effect, and Catalyst continues to be a third-party beneficiary of Section 5.11(b) (Noncompetition) of the SPA.

1.3. The License Agreement continues in full force and effect in accordance with its terms, including without limitation, Section 15.14 (Non Compete) of the License Agreement, and continues to be an agreement between BioMarin and Catalyst.

2. SETTLEMENT, DISCLAIMER, AND RELEASE

2.1. **Settlement**. The Parties agree that by entering into this Agreement they are resolving all outstanding disputes concerning the Parties' respective obligations related to the SPA Milestones and/or SPA Milestone Payments described in Sections 1.4(g) and/or 1.4(h) of the SPA that have arisen since the beginning of time through the Effective Date and will take all necessary steps to stop and abandon any presently existing efforts to continue with any such disputes. For the avoidance of doubt, this Agreement does not limit the rights of the Stockholders to enforce this Agreement against Catalyst if the milestones set forth in Section 3 of this Agreement are met and the related payments set forth in Section 3 of this Agreement are not made when due. For the avoidance of doubt, BioMarin shall not be liable to any other Party or Third Party, and no other Party or Third Party shall seek recourse against BioMarin, for any matter relating to, based upon, or arising out of the milestones and milestone payments set forth in Section 3 of this Agreement. For purposes of this Agreement, "**Third Party**" means any Person other than a Party or an Affiliate of a Party.

2.2. **Disclaimer.** Nothing contained in this Agreement, nor any actions taken by any Party in connection with this Agreement, shall constitute, be construed as, or be deemed to be an admission of any kind whatsoever on the part of any of the Parties. Each of the Parties expressly denies any fault, wrongdoing, or liability to each other and, by entering into this Agreement, intends to avoid potential litigation with respect to the matters covered by this Agreement.

2.3. Releases.

2.3.1. Definitions. For purposes of this Agreement, "**Affiliate**" means, with respect to a Party, any Person that controls, is controlled by, or is under common control with that Party. For the purpose of this definition, "control" will refer to: (a) the possession, directly or indirectly, of the power to direct the management or policies of an entity, whether through the ownership of voting securities, by contract or otherwise; or (b) the ownership, directly or indirectly, of 50% or more of the voting securities of such entity. For purposes of this Agreement, "**Person**" means an individual, corporation, partnership, limited liability company, trust, business trust, association, joint stock company, joint venture, pool, syndicate, sole proprietorship, unincorporated organization, governmental authority or any other form of entity not specifically listed herein.

2.3.2. Release of Certain Claims by Aceras and the Stockholders in Favor of BioMarin and Catalyst. For and in consideration of the agreements set forth herein, Aceras, on its own behalf and on behalf of the Stockholders, for itself, for the Stockholders, for their Affiliates, and for each of its present and former directors, officers, shareholders, members, managers, agents, attorneys, and successors, and anyone claiming by or through it, (the "**Huxley Parties**") do herewith now and forever absolutely, unconditionally and irrevocably release, indemnify and discharge BioMarin and Catalyst, and each of their present and former Affiliates, directors, officers, shareholders, members, managers, agents, attorneys, and successors, and anyone claiming by or through them (all of the foregoing being referred to as the "**BioMarin Released Parties**" and the "**Catalyst Released Parties**" respectively) from any and all claims, demands, rights, actions, suits, proceedings, liabilities, obligations and causes of action of any kind and nature whatsoever, fixed or contingent, known or unknown, liquidated or unliquidated, that any of the Huxley Parties ever had, now have or hereafter may possibly have, against the BioMarin Released Parties and the Catalyst Released Parties based upon or by reason of any matter, cause or thing resulting from, arising out of or incurred with respect to, or alleged to result from, arise out of or be incurred with respect to, acts or omissions to act of any nature and kind whatsoever by any of the BioMarin Released Parties or the Catalyst Released Parties that both (a) occurred, in whole or in part, prior to or as of the Effective Date of this Agreement, and (b) are based upon, or arise out of, the SPA Milestones and/or SPA Milestone Payments described in Sections 1.4(g) and/or 1.4(h) of the SPA (the "**Huxley Released Claims**"). For the avoidance of doubt, the following claims are not released hereunder: (x) claims that are not based upon, or do not arise out of, the SPA Milestones and/or SPA Milestone Payments described in Sections 1.4(g) and/or 1.4(h) of the SPA, and (y) claims for enforcement of the terms of this Agreement. Each of the Huxley Parties releases and discharges the BioMarin Released Parties and the Catalyst Released Parties from any and all claims that relate to the Huxley Released Claims, and they

specifically waive any right to become, and promise not to become, a member of any class in any proceeding or case in which a claim or claims that relate to the Huxley Released Claims is asserted against the BioMarin Released Parties or the Catalyst Released Parties, arising, in whole or in part, from any event which occurred as of and through the date of this Agreement. If any of the Huxley Parties violate this Agreement by instituting a lawsuit or other action that relates to the Huxley Released Claims against the BioMarin Released Parties or the Catalyst Released Parties (other than a lawsuit to enforce this Agreement), each of the Huxley Parties, jointly and severally, agrees that it will pay all reasonable costs and expenses of defending against the suit incurred by the BioMarin Released Parties or the Catalyst Released Parties, including reasonable attorney's fees. Each of the Huxley Parties agrees that it will not assert or file any claim, complaint, charge, suit, or action against any of the parties they have released hereunder relating to or arising out of any of the Huxley Released Claims. In the event that any claim, complaint, charge, suit, or action is asserted or filed in breach of this section (collectively, an "**Aceras Claim-In-Breach**"), each affected BioMarin Released Party or Catalyst Released Party shall be entitled to deliver this Agreement to the court in which such suit has been brought seeking a dismissal of such suit, and it will be a bar to such suit. Further, the party that has brought an Aceras Claim-In-Breach shall be obligated to reimburse the BioMarin Released Party or Catalyst Released Party against whom such suit has been brought in violation of this section for all reasonable costs and attorney's fees that the BioMarin Released Party or the Catalyst Released Party incurs in investigating and defending against such Aceras Claim-In-Breach, whether incurred by such BioMarin Released Party or Catalyst Released Party prior to the filing of any claim in any tribunal, or prior to any trial or hearing, or at trial, or on appeal. The Huxley Parties acknowledge that they may hereafter discover facts different from, or in addition to, those which such party now believes to be true with respect to any and all of the Huxley Released Claims, and no such additional fact shall affect the validity or enforceability of this Agreement and the release contemplated hereby.

2.3.3. Release of Certain Claims by Catalyst in Favor of BioMarin and the Stockholders. For and in consideration of the agreements set forth herein, Catalyst, for itself and its Affiliates, and for each of its present and former directors, officers, shareholders, members, managers, agents, attorneys, and successors, and anyone claiming by or through it, (the "**Catalyst Parties**") does herewith now and forever absolutely, unconditionally and irrevocably release, indemnify and discharge the BioMarin Released Parties and the Stockholders, and each of the Stockholders' present and former Affiliates, directors, officers, shareholders, members, managers, agents, attorneys, and successors, and anyone claiming by or through them (all of the foregoing being referred to as the "**Huxley Released Parties**") from any and all claims, demands, rights, actions, suits, proceedings, liabilities, obligations and causes of action of any kind and nature whatsoever, fixed or contingent,

known or unknown, liquidated or unliquidated, that any of the Catalyst Parties ever had, now have or hereafter may possibly have, against the BioMarin Released Parties and the Huxley Released Parties based upon or by reason of any matter, cause or thing resulting from, arising out of or incurred with respect to, or alleged to result from, arise out of or be incurred with respect to, acts or omissions to act of any nature and kind whatsoever by any of the BioMarin Released Parties or the Huxley Released Parties that both (a) occurred, in whole or in part, prior to or as of the Effective Date of this Agreement, and (b) are based upon, or arise out of, the SPA Milestones and/or SPA Milestone Payments described in Sections 1.4(g) and/or 1.4(h) of the SPA (the "**Catalyst Released Claims**"). For the avoidance of doubt, the following claims are not released hereunder: (x) claims that are not based upon, or do not arise out of, the SPA Milestones and/or SPA Milestone Payments described in Sections 1.4(g) and/or 1.4(h) of the SPA, and (y) claims for enforcement of the terms of this Agreement. Each of the Catalyst Parties releases and discharges the BioMarin Released Parties and the Huxley Released Parties from any and all claims that relate to the Catalyst Released Claims, and they specifically waive any right to become, and promise not to become, a member of any class in any proceeding or case in which a claim or claims that relate to the Catalyst Released Claims is asserted against the BioMarin Released Parties or the Huxley Released Parties, arising, in whole or in part, from any event which occurred as of and through the date of this Agreement. If any of the Catalyst Parties violate this Agreement by instituting a lawsuit or other action that relates to the Catalyst Released Claims against the BioMarin Released Parties or the Huxley Released Parties (other than a lawsuit to enforce this Agreement), each of the Catalyst Parties, jointly and severally, agrees that it will pay all reasonable costs and expenses of defending against the suit incurred by the BioMarin Released Parties or the Huxley Released Parties, including reasonable attorney's fees. Each of the Catalyst Parties agrees that it will not assert or file any claim, complaint, charge, suit, or action against any of the parties they have released hereunder relating to or arising out of any of the Catalyst Released Claims. In the event that any claim, complaint, charge, suit, or action is asserted or filed in breach of this section (collectively, a "**Catalyst Claim-In-Breach**"), each affected BioMarin Released Party or Huxley Released Party shall be entitled to deliver this Agreement to the court in which such suit has been brought seeking a dismissal of such suit, and it will be a bar to such suit. Further, the party that has brought a Catalyst Claim-In-Breach shall be obligated to reimburse the BioMarin Released Party or Huxley Released Party against whom such suit has been brought in violation of this section for all reasonable costs and attorney's fees that the BioMarin Released Party or the Huxley Released Party incurs in investigating and defending against such Catalyst Claim-In-Breach, whether incurred by such BioMarin Released Party or Huxley Released Party prior to the filing of any claim in any tribunal, or prior to any trial or hearing, or at trial, or on appeal. The Catalyst Parties

acknowledge that they may hereafter discover facts different from, or in addition to, those which such party now believes to be true with respect to any and all of the Catalyst Released Claims, and no such additional fact shall affect the validity or enforceability of this Agreement and the release contemplated hereby.

2.3.4. Release of Certain Claims by BioMarin in Favor of Catalyst and the Stockholders. For and in consideration of the agreements set forth herein, BioMarin, for itself and its Affiliates, and for each of its present and former directors, officers, shareholders, members, managers, agents, attorneys, and successors, and anyone claiming by or through it, (the "**BioMarin Parties**") does herewith now and forever absolutely, unconditionally and irrevocably release, indemnify and discharge the Catalyst Released Parties and the Huxley Released Parties from any and all claims, demands, rights, actions, suits, proceedings, liabilities, obligations and causes of action of any kind and nature whatsoever, fixed or contingent, known or unknown, liquidated or unliquidated, that any of the BioMarin Parties ever had, now have or hereafter may possibly have, against the Catalyst Released Parties and the Huxley Released Parties based upon or by reason of any matter, cause or thing resulting from, arising out of or incurred with respect to, or alleged to result from, arise out of or be incurred with respect to, acts or omissions to act of any nature and kind whatsoever by any of the Catalyst Released Parties or the Huxley Released Parties that both (a) occurred, in whole or in part, prior to or as of the Effective Date of this Agreement, and (b) are based upon, or arise out of, the SPA Milestones and/or SPA Milestone Payments described in Sections 1.4(g) and/or 1.4(h) of the SPA (the "**BioMarin Released Claims**"). For the avoidance of doubt, the following claims are not released hereunder: (x) claims that are not based upon, or do not arise out of, the SPA Milestones and/or SPA Milestone Payments described in Sections 1.4(g) and/or 1.4(h) of the SPA, and (y) claims for enforcement of the terms of this Agreement. Each of the BioMarin Parties releases and discharges the Catalyst Released Parties and the Huxley Released Parties from any and all claims that relate to the BioMarin Released Claims, and they specifically waive any right to become, and promise not to become, a member of any class in any proceeding or case in which a claim or claims that relate to the BioMarin Released Claims is asserted against the Catalyst Released Parties or the Huxley Released Parties, arising, in whole or in part, from any event which occurred as of and through the date of this Agreement. If any of the BioMarin Parties violate this Agreement by instituting a lawsuit or other action that relates to the BioMarin Released Claims against the Catalyst Released Parties or the Huxley Released Parties (other than a lawsuit to enforce this Agreement), each of the BioMarin Parties, jointly and severally, agrees that it will pay all reasonable costs and expenses of defending against the suit incurred by the Catalyst Released Parties or the Huxley Released Parties, including reasonable attorney's fees. Each of the BioMarin Parties agrees that it will not assert or file any claim, complaint, charge, suit, or action against any of the parties they have

released hereunder relating to or arising out of any of the BioMarin Released Claims. In the event that any claim, complaint, charge, suit, or action is asserted or filed in breach of this section (collectively, a "**BioMarin Claim-In-Breach**"), each affected Catalyst Released Party or Huxley Released Party shall be entitled to deliver this Agreement to the court in which such suit has been brought seeking a dismissal of such suit, and it will be a bar to such suit. Further, the party that has brought a BioMarin Claim-In-Breach shall be obligated to reimburse the Catalyst Released Party or Huxley Released Party against whom such suit has been brought in violation of this section for all reasonable costs and attorney's fees that the Catalyst Released Party or Huxley Released Party incurs in investigating and defending against such BioMarin Claim-In-Breach, whether incurred by such Catalyst Released Party or Huxley Released Party prior to the filing of any claim in any tribunal, or prior to any trial or hearing, or at trial, or on appeal. The BioMarin Parties acknowledge that they may hereafter discover facts different from, or in addition to, those which such party now believes to be true with respect to any and all of the BioMarin Released Claims, and no such additional fact shall affect the validity or enforceability of this Agreement and the release contemplated hereby.

3. CATALYST MILESTONES

3.1. Milestone Payments. Catalyst shall directly pay to the Stockholders any payment that may become due to the Stockholders pursuant to the provisions of this Section 3.1 within thirty (30) days of the date such payment becomes due as provided herein, and each Stockholder shall receive only that portion of each contingent payment that is the product of (A) the aggregate amount payable to the Stockholders, and (B) the earnout percentage set forth opposite such Stockholder's name on Exhibit 3.2 to this Agreement. All payments to the Stockholders under this Section 3.1 are one-time payments, and once a payment is triggered under a subsection of this Section 3.1, no further or other payment shall be triggered under such subsection, regardless of the number of times the described event occurs. For the sake of clarity, in no event will the total amount payable under this Section 3.1 exceed an aggregate of Two Million Dollars (US $2,000,000).

3.1.1. Execution. Upon execution of this Agreement, Catalyst shall pay to the Stockholders an aggregate of One Million Dollars (US $1,000,000).

3.1.2. NDA Approval. Upon first receipt of an approval by Catalyst or any Affiliate or Sublicensee (as such term is defined in the SPA) of Catalyst of an NDA (as such term is defined in the SPA) for a Product (as such term is defined in the SPA), Catalyst shall pay to the Stockholders an aggregate of One Million Dollars (US $1,000,000).

4. REPRESENTATIONS, WARRANTIES AND COVENANTS

4.1. Representations and Warranties by Each Party. Each Party represents and warrants, and covenants, as applicable, to the other Parties as of the Effective Date that:

(a) *Organization; Power and Authority*. Such Party (i) is duly organized, validly existing and in good standing under the laws of its jurisdiction of organization and (ii) has the power, authority and legal right, and is free to enter into this Agreement and, in so doing, will not violate any other agreement to which such Party is a party as of the Effective Date, or conflict with the rights granted to any Third Party;

(b) *Due Execution*. This Agreement has been duly executed and delivered on behalf of such Party and constitutes a legal, valid, and binding obligation of such Party and is enforceable against it in accordance with its terms, subject to the effects of bankruptcy, insolvency, or other laws of general application affecting the enforcement of creditor rights and judicial principles affecting the availability of specific performance and general principles of equity;

(c) *Authorization*. Such Party has taken all action necessary to authorize the execution and delivery of this Agreement;

(d) *Consents*. Such Party has obtained all necessary consents, approvals, and authorizations of all regulatory authorities and other Third Parties required to be obtained by such Party in connection with the execution and delivery of this Agreement and the performance of its obligations hereunder;

(e) *No Litigation.* There is no action or proceeding pending or, to the knowledge of such Party, threatened that could reasonably be expected to impair or delay the ability of such Party to perform its obligations under this Agreement; and

(f) *No Conflicts*. The execution and delivery of this Agreement and the performance of such Party's obligations hereunder (i) do not and will not conflict with or violate any requirement of Applicable Law or any provision of the articles of incorporation, bylaws, limited partnership agreement, or any similar instrument of such Party, as applicable, in any material way, and (ii) do not and will not conflict with, violate or breach, or constitute a default or require any consent under, any contractual obligation or court or administrative order by which such Party is or will be bound.

4.2. Representations and Warranties by Aceras. Aceras represents and warrants to each of BioMarin and Catalyst as of the Effective Date that:

(a) *Authority to Bind the Stockholders*. Aceras has the power, authority and legal right to bind the Stockholders pursuant to any section of this Agreement under which Aceras agrees to anything on behalf of the Stockholders.

(b) *No Withholding Required*. No U.S. federal income tax withholding is required for the payment that Catalyst is required to make to the Stockholders under Section 3.1.1 of this Agreement. Further, the Stockholders shall provide Catalyst with updated W-9s and W-8BENs, as applicable, if and when a second payment becomes due to the Stockholders under Section 3.1.2 of this Agreement. In that regard, Aceras agrees, if it is asserted by the Internal Revenue Service ("IRS") that Catalyst was obligated to withhold on any payments made under Section 3.1 of this Agreement and to remit such amounts to the IRS, it will indemnify Catalyst for any amounts that Catalyst becomes obligated to remit to the IRS (including any applicable interest and penalties) relating to such payments.

5. TERM

5.1. Term. The term of this Agreement will commence as of the Effective Date and will continue indefinitely.

6. GENERAL PROVISIONS

6.1. Assignment. No Party may assign its rights and obligations under this Agreement without the prior written consent of the other Parties, except that: (a) any Party may assign its rights and obligations under this Agreement in whole or in part to one or more of its Affiliates without the consent of the other Parties; and (b) any Party may assign this Agreement in connection with a change of control transaction or sale of substantially all the assets to which this Agreement relates; provided, that any such permitted assignee assumes all obligations of its assignor under this Agreement. The assigning Party will provide the other Parties with prompt written notice of any such assignment. No permitted assignment will relieve the assignor of liability for its obligations hereunder. Any attempted assignment in contravention of the foregoing will be void.

6.2. Severability. Should one or more of the provisions of this Agreement become void or unenforceable as a matter of law, then such provision will be ineffective only to the extent of such prohibition or invalidity, without invalidating the remainder of this Agreement, and the Parties agree to substitute a valid and enforceable provision therefor which, as nearly as possible, achieves the desired economic effect and mutual understanding of the Parties under this Agreement.

6.3. Governing Law; Disputes.

6.3.1. Governing Law. This Agreement will be governed by and construed under the laws in effect in the State of New York, United States of America, without giving effect to any conflicts of laws provision thereof or of any other jurisdiction that would produce a contrary result.

6.3.2. Disputes.

(a) *Arbitration*. Any dispute arising under this Agreement or other legal proceeding relating to this Agreement or the enforcement of any provision of this Agreement shall be finally resolved by binding arbitration administered by JAMS pursuant to JAMS Streamlined Arbitration Rules and Procedures then in effect (the "**JAMS Rules**"), and judgment on the arbitration award may be entered in any court having jurisdiction thereof. The arbitration shall be conducted by a single, neutral arbitrator who shall have experience with respect to the matter(s) to be arbitrated. If, within thirty (30) days after initiation of arbitration, the Parties are unable to agree on a single arbitrator, the arbitrator shall be appointed by JAMS. The place of arbitration shall be New York City, New York. Any Party may apply to the arbitrator for interim injunctive relief until the arbitration award is rendered or the controversy is otherwise resolved. Nothing contained herein shall be construed to permit the arbitrator to award punitive, exemplary or similar damages. Each Party shall bear its own costs and expenses and attorneys' fees and an equal share of the arbitrators' fees and any administrative fees of arbitration. Except to the extent necessary to confirm an award or as may be required by law, neither a Party nor an arbitrator may disclose the existence, content, or results of an arbitration without the prior written consent of all Parties. In no event shall an arbitration be initiated after the date when commencement of a legal or equitable proceeding based on the dispute, controversy or claim would be barred by the applicable New York statute of limitations. The Parties further agree that any payments made pursuant to this Agreement pending resolution of the dispute shall be refunded if the arbitrator determines that such payments are not due.

6.4. Waivers and Amendments. The failure of any Party to assert a right hereunder or to insist upon compliance with any term or condition of this Agreement will not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition by the other Parties. No waiver will be effective unless it has been given in writing and signed by the Party giving such waiver. No provision of this Agreement may be amended or modified other than by a written document signed by authorized representatives of each Party.

6.5. Relationship of the Parties. Nothing contained in this Agreement will be deemed to constitute a partnership, joint venture, or legal entity of any type between the Parties, or to constitute one Party as the agent of the other. Moreover, each Party agrees not to construe this Agreement, or

any of the transactions contemplated hereby, as a partnership for any tax purposes. Each Party will act solely as an independent contractor, and nothing in this Agreement will be construed to give any Party the power or authority to act for, bind, or commit the other Parties.

6.6. **Successors and Assigns**. This Agreement will be binding upon and inure to the benefit of the Parties hereto and their respective successors and permitted assigns.

6.7. **Notices**. All notices, consents, waivers, and other communications under this Agreement must be in writing and will be deemed to have been duly given when: (a) delivered by hand (with written confirmation of receipt); or (b) when received by the addressee, if sent by an internationally recognized overnight delivery service (receipt requested), in each case to the appropriate addresses set forth below (or to such other addresses as a Party may designate by written notice):

If to Aceras:

Aceras BioMedical, LLC
325 East 41st Street, Suite 107
New York, NY 10017
Attention: Matthew Wyckoff, M.D.

If to BioMarin:

BioMarin Pharmaceutical, Inc.
105 Digital Drive
Novato, Ca 94949
Attention: G. Eric Davis, Esq., EVP & General Counsel

If to Catalyst:

Catalyst Pharmaceuticals, Inc.
355 Alhambra Circle, Suite 1250
Coral Gables, FL 33131
Attention: Patrick J McEnany, CEO

6.8. **No Third Party Beneficiary Rights**. Except as expressly provided in this Agreement, this Agreement is not intended to and will not be construed to give any Third Party any interest or rights (including, without limitation, any Third Party beneficiary rights) with respect to or in connection with any agreement or provision contained herein or contemplated hereby.

6.9. Entire Agreement. This Agreement sets forth the entire agreement and understanding of the Parties as to the subject matter hereof and supersede all proposals, oral or written, and all other prior communications between the Parties with respect to such subject matter.

6.10. Headings. The headings to the Sections hereof are not a part of this Agreement, but are merely for convenience to assist in locating and reading the several Sections hereof.

6.11. Interpretation. Except where the context expressly requires otherwise, (a) the use of any gender herein will be deemed to encompass references to either or both genders, and the use of the singular will be deemed to include the plural (and vice versa), (b) the words "include", "includes" and "including" will be deemed to be followed by the phrase "without limitation", (c) the word "will" will be construed to have the same meaning and effect as the word "shall," (d) any definition of or reference to any agreement, instrument or other document herein will be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein), (e) any reference herein to any Person will be construed to include the Person's successors and permitted assigns, (f) the words "herein", "hereof" and "hereunder," and words of similar import, will be construed to refer to this Agreement in its entirety and not to any particular provision hereof, (g) unless otherwise specified, all references herein to Sections will be construed to refer to Sections of this Agreement, (h) the word "notice" means notice in writing (whether or not specifically stated) and will include notices, consents, approvals and other written communications contemplated under this Agreement, (i) provisions that require that a Party, the Parties or any committee hereunder "agree," "consent" or "approve" or the like will require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise (but excluding e-mail and instant messaging), (j) references to any specific law, rule or regulation, or article, section or other division thereof, will be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof and (k) the term "or" will be interpreted in the inclusive sense commonly associated with the term "and/or."

6.12. Cumulative Remedies. No remedy referred to in this Agreement is intended to be exclusive, but each will be cumulative and in addition to any other remedy referred to in this Agreement or otherwise available under law.

6.13. Waiver of Rule of Construction. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this Agreement. Accordingly, any rule of construction that any ambiguity in this Agreement will be construed against the drafting Party will not apply.

6.14. Counterparts. The Agreement may be executed in two or more counterparts, including by facsimile or PDF signature pages, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

[*Signatures on next page*]

IN WITNESS WHEREOF, the Parties intending to be bound have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

ACERAS BIOMEDICAL, LLC
On Behalf of Itself and for All Stockholders

By: /s/ John Liatos

Name: John Liatos

Title: Member

Date: July 27, 2018

BIOMARIN PHARMACEUTICAL INC.

By: /s/ G. Eric Davis

Name: G. Eric Davis

Title: EVP, General Counsel

Date: July 26, 2018

CATALYST PHARMACEUTICALS, INC.

By: /s/ Patrick J. McEnany

Name: Patrick J. McEnany

Title: Chairman, President and CEO

Date: July 27, 2018

EXHIBIT 3.2

Stockholder Name	Earnout Percentage
Aceras BioMedical, LLC	92.5%
Richard Stewart	5.25%
Anthony Clarke	2.25%
TOTALS:	100.00%

Exhibit 31.1

Certification of Principal Executive Officer

I, Patrick J. McEnany, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Catalyst Pharmaceuticals, Inc.;

2. Based on my knowledge, this quarterly report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiary, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):

   a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2018

/s/ Patrick J. McEnany
Patrick J. McEnany
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

Certification of Principal Financial Officer

I, Alicia Grande, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Catalyst Pharmaceuticals, Inc.;

2. Based on my knowledge, this quarterly report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15(d)-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiary, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's board of directors (or persons performing the equivalent functions):

   a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2018

/s/ Alicia Grande

Alicia Grande
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

Certification Required by 18 U.S.C. Section 1350
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)

I, Patrick J. McEnany as Principal Executive Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Quarterly Report on Form 10-Q of the Company for the quarterly period ended June 30, 2018 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 7, 2018

/s/ Patrick J. McEnany
Patrick J. McEnany
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

Certification Required by 18 U.S.C. Section 1350
(as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002)

I, Alicia Grande as Principal Financial Officer of Catalyst Pharmaceuticals, Inc. (the "Company"), certify, pursuant to 18 U.S.C. Section 1350 (as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002), that to my knowledge:

1. the accompanying Quarterly Report on Form 10-Q of the Company for the quarterly period ended June 30, 2018 (the "Report"), filed with the U.S. Securities and Exchange Commission, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 7, 2018

/s/ Alicia Grande
Alicia Grande
Chief Financial Officer
(Principal Financial Officer)